

CIRM approves \$27 million for initiatives to accelerate promising stem cell research projects

Posted: December 8, 2011

Los Angeles, Calif. – The California Institute for Regenerative Medicine, the state stem cell agency, today enhanced its efforts to proactively discover and fund innovative research and to provide continuous support to successful research programs, speeding the development of new stem cell-based therapies.

The governing board's support of a \$15 million External Innovation Initiative responds to a call by the external advisory panel that reviewed CIRM last year. The panel recommended "...a more aggressively proactive approach to identifying innovative projects across the stem cell therapeutic landscape that shows promise for moving into translational research, clinical trials and product development."

The External Innovation Initiative, which is part of the agency's Opportunity Funds, allows CIRM to identify outstanding research taking place within the jurisdiction of any of the agency's external collaboration network of funding partners. These partners, including 12 countries, two international states, one domestic state, two foundations, and most recently CIRM's collaborative relationship with the National Institutes of Health, are available on the CIRM website. CIRM can fund new or supplement an existing award to a California researcher who forms a partnership with the external scientist. The external portion of the award would be supported by the funding partner.

"This initiative allows CIRM to support ground-breaking research wherever it is taking place," said CIRM president Alan Trounson. "With our extensive network of collaborations and collaborative funding partners, we can jointly fund the research taking place outside California, with CIRM promoting collaborations with researchers who can add scientific expertise and accelerate the work taking place inside California."

In addition to the External Innovation Initiative, the board approved the final piece of the Opportunity Fund, called the Bridging Fund. This \$12 million initiative will provide supplemental funding for the most promising existing CIRM-funded projects to enable the research to continue without interruption until next applicable round of CIRM funding or receipt of other funds. Together, the three pieces of the Opportunity Fund (the first of which helps create industry partnerships and was approved at the previous board meeting) allow CIRM to become more nimble in working with industry, promoting great science wherever it is taking place, and providing a continuous source of funding for successful research.

"CIRM plays a critical role by funding research from the basic discovery phase through the research to develop therapeutic candidates and the preclinical studies to help assure that the candidate approach is safe for patients in early phase clinical trials," said Jonathan Thomas, Chair of the CIRM governing board. "This program will allow CIRM to seamlessly fund projects that are making good progress toward clinical trials, and to give researchers the stable source of funding they need to focus on developing therapies. This more seamless approach to funding will accelerate our ability to move high quality projects forward and more urgently address the needs of patient who are looking to CIRM for new therapies."

Innovative funding arrangements and engagement with industry, such as those supported by the three Opportunity Fund initiatives, are a key component of the 2012 strategic plan update. At the meeting, board members heard an update on the 2012 strategic plan. The update builds on the initial 2006 strategic plan, a 2009/2010 revision, and incorporates recommendations from a 2010 external review panel. The proposed update includes input from stakeholders gathered over the past two months, including meetings with the public, industry, patient advocates and collaborative funding partners. The recurring themes were the need to raise public awareness of CIRM's activities, the need to reach a major therapeutic milestone, and the need for greater engagement and interaction with industry. The board will weigh in on a draft of the strategic plan at the January meeting. (The two previous strategic plans are available on the CIRM website.)

The governing board also voted on two new research initiatives during the meeting, and approved the third Research Leadership Award. The award will aid recruiting Zhigang He from Children's Hospital Boston to the University of California Berkeley. The funds are part of a recruitment package, but won't be dispersed until He's recruitment is finalized. It is the third Research Leadership award to be approved.

This \$5.6 million award over six years will fund He's research into restoring neural function after spinal cord injury. His work focuses on

regenerating the nerve projections that carry signals up and down the spine. These projections, called axons, are often damaged during a spinal cord injury, preventing them from carrying signals. He intends to develop stem cell technologies to bridge the site of the injury and restore the ability for nerve signals to travel along the spine.

The board also approved a concept proposal for an initiative to create a new stem cell bank. In recent years, human pluripotent stem cells (both embryonic stem cells and reprogrammed iPS cells) are emerging as excellent models for disease. Cells reprogrammed from people with Parkinson's disease, for example, can mimic the disease in a lab dish and allow scientists to test drugs and develop therapies.

In order to create useful cell lines for researchers to use in disease therapy research, CIRM has proposed creating a bank of cells carrying disease mutations and making those cells available to researchers in California and worldwide. The first part of this initiative is already in place, with the CIRM/NIH collaboration to generate cell lines from people with Huntington's disease, Parkinson's disease and Amyotrophic Lateral Sclerosis.

The CIRM Human Pluripotent Stem Cell Initiative, consisting of three RFA concepts and approved today, would fund:

1. Collection of samples from people with particular prevalent diseases, with the goal of deriving lines from 1,200 individuals. The resulting lines would be available free of charge to the research groups who acquired the samples. This RFA would be worth up to \$4 million
2. Derivation of reprogrammed pluripotent cell lines (iPS cells) from the samples. This award, worth up to \$16 million, would go to a single organization that can derive the lines under consistent conditions.
3. Creation of a pluripotent stem cell bank containing the iPS cells created from the first two initiatives and also embryonic stem cell lines and iPS cell lines already created by California researchers. This bank would make well characterized, disease-specific stem cell lines available to researchers worldwide. The award would be worth up to \$10 million over three years.

The RFAs would be available in May 2012, with the ICOC voting on final applications early 2013.

About CIRM: CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided \$3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research opportunities. A list of grants and loans awarded to date may be seen here: <http://www.cirm.ca.gov/for-researchers/researchfunding>

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